(19) World Intellectual Property Organization

International Bureau





(43) International Publication Date 25 August 2005 (25.08.2005)

PCT

(10) International Publication Number WO 2005/078097 A2

(51) International Patent Classification7:

(21) International Application Number:

PCT/US2005/004270

C12N 15/11

(22) International Filing Date: 9 February 2005 (09.02.2005)

(25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

60/543,480 10 February 2004 (10.02 2004) US PCT/US04/016390 24 May 2004 (24.05 2004) US

- (71) Applicant (for all designated States except US): SIRNA THERAPEUTICS, INC. [US/US]; 2950 Wilderness Place, Boulder, CO 80301 (US)
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): JADHAV, Vasant [IN/US]; 4735 Lucca Drive, Longmont, CO 80503 (US) ZINNEN, Shawn [US/US]; 2378 Birch Street, Denver, CO 80207 (US).
- (74) Agent: GREENFIELD, Michael, S.; McDonnell Boehnen Hulbert and Berghoff, 300 South Wacker Drive, Suite 3200, Chicago, IL 60606 (US).

- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

 without international search report and to be republished upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette

(54) Title: RNA INTERFERENCE MEDIATED INHIBITION OF GENE EXPRESSION USING MULTIFUNCTIONAL SHORT INTERFERING NUCLEIC ACID (Multifunctional siNA)

(57) Abstract: [00101] The present invention concerns methods and nucleic acid based reagents useful in modulating gene expression in a variety of applications, including use in therapeutic, veterinary, agricultural, diagnostic, target validation, and genomic discovery applications. Specifically, the invention relates to multifunctional short interfering nucleic acid (multifunctional siNA) molecules that modulate the expression of one or more genes in a biologic system, such as a cell, tissue, or organism via RNA interference (RNAi). The bifuntional short interfering nucleic acid (multifunctional siNA) molecules of the invention can target more than one regions of nucleic acid sequence in a single target nucleic acid molecule or can target regions of nucleic acid sequence in differing target nucleic acid molecules. The self multifunctional siNA molecules are useful in the treatment of any disease or condition that responds to modulation of gene expression or activity in a cell, tissue, or organism.

